

(19) World Intellectual Property  
Organization  
International Bureau



(43) International Publication Date  
13 October 2005 (13.10.2005)

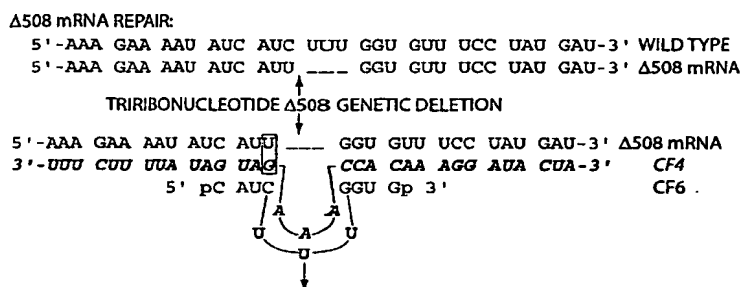
PCT

(10) International Publication Number  
**WO 2005/094370 A2**

- (51) International Patent Classification: Not classified
- (21) International Application Number: PCT/US2005/010744
- (22) International Filing Date: 29 March 2005 (29.03.2005)
- (25) Filing Language: English
- (26) Publication Language: English
- (30) Priority Data:  
60/557,732 29 March 2004 (29.03.2004) US  
60/560,026 7 April 2004 (07.04.2004) US
- (63) Related by continuation (CON) or continuation-in-part (CIP) to earlier applications:  
US 60/557,732 (CIP)  
Filed on 29 March 2004 (29.03.2004)  
US 60/560,026 (CIP)  
Filed on 7 April 2004 (07.04.2004)
- (71) Applicant (for all designated States except US): THE GENERAL HOSPITAL CORPORATION [US/US]; 55 Fruit Street, Boston, MA 02114 (US).
- (72) Inventors; and
- (75) Inventors/Applicants (for US only): TABATADZE, David, R. [RU/US]; 16 Simmons Avenue, Worcester, MA 01605 (US). ZAMECNIK, Paul, C. [US/US]; 101 Chestnut Street, Boston, MA 02108 (US). RAY-CHOWDHURY, Malay, K. [US/US]; 40 Bartlett Avenue, Lexington, MA 02420 (US). CANTIello, Horacio, F. [US/US]; 237 Wethersfield Street, Rowley, MA 01969 (US).
- (74) Agent: ELRIFI, Ivor, R.; Mintz, Levin, Cohn, Ferris, Glovsky and PopeoPC, One Financial Center, Boston, MA 02111 (US).
- (81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI,

[Continued on next page]

(54) Title: OLIGONUCLEOTIDE COMPLEX COMPOSITIONS AND METHODS OF USE AS GENE ALTERATION TOOLS



SCHEME 1

A

(57) Abstract: Compositions and methods of treatments of cells are provided for altering the phenotype of a cell by administering an oligonucleotide complex to the cell, the complex having two strands and chemical modifications.

VARIED RIBONUCLEOTIDE INSERTION AND DELETION STEPS, WITH (c) BEING THE BEST RESULT:

REPEATED SEQUENCE ANALYSIS OF RT-PCR PRODUCTS WITH DIFFERENT PRIMERS:

(a) ARMS REVERSE PRIMER (N1)

5' -AAA GAA AAU AUC A --- UGU GGU GUU UCC UAU GAU-3'

(b) ARMS FORWARD PRIMER (NF1)

5' -AAA GAA AAU AUC AUC UGU --- GUU UCC UAU GAU-3'

(c) ARMS FORWARD PS PRIMER (SNF1)

5' -AAA GAA AAU AUC AUC UGU GGU GUU UCC UAU GAU-3'

SCHEME 1

B

WO 2005/094370 A2



GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SM, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US (patent), UZ, VC, VN, YU, ZA, ZM, ZW.

FR, GB, GR, HU, IE, IS, IT, LT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

**Published:**

— without international search report and to be republished upon receipt of that report

(84) **Designated States** (*unless otherwise indicated, for every kind of regional protection available*): ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI,

*For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.*